(c) Where a drug has been designated as an orphan drug because the prevalence of a disease or condition (or, in the case of vaccines, diagnostic drugs, or preventive drugs, the target population) is under 200,000 in the United States at the time of designation, its designation will not be revoked on the ground that the prevalence of the disease or condition (or the target population) becomes more than 200,000 persons.

(d) If FDA revokes orphan-drug designation, FDA will publicize that the drug is no longer designated in accordance with § 316.28(e).

[57 FR 62085, Dec. 29, 1992, as amended at 78 FR 35134, June 12, 2013]

§ 316.30 Annual reports of holder of orphan-drug designation.

Within 14 months after the date on which a drug was designated as an orphan drug and annually thereafter until marketing approval, the sponsor of a designated drug shall submit a brief progress report to the FDA Office of Orphan Products Development on the drug that includes:

(a) A short account of the progress of drug development including a review of preclinical and clinical studies initiated, ongoing, and completed and a short summary of the status or results of such studies.

(b) A description of the investigational plan for the coming year, as well as any anticipated difficulties in development, testing, and marketing; and

(c) A brief discussion of any changes that may affect the orphan-drug status of the product. For example, for products nearing the end of the approval process, sponsors should discuss any disparity between the probable marketing indication and the designated indication as related to the need for an amendment to the orphan-drug designation pursuant to § 316.26.

Subpart D—Orphan-drug Exclusive Approval

§ 316.31 Scope of orphan-drug exclusive approval.

(a) FDA may approve a sponsor's marketing application for a designated orphan drug for use in the rare disease or condition for which the drug was designated, or for select indication(s) or use(s) within the rare disease or condition for which the drug was designated. Unless FDA previously approved the same drug for the same use or indication, FDA will not approve another sponsor's marketing application for the same drug for the same use or indication before the expiration of 7 years from the date of such approval as stated in the approval letter from FDA, except that such a marketing application can be approved sooner if, and at such time as, any of the following occurs:

(1) Withdrawal of exclusive approval or revocation of orphan-drug designation by FDA under any provision of this part; or

(2) Withdrawal for any reason of the marketing application for the drug in question; or

(3) Consent by the holder of exclusive approval to permit another marketing application to gain approval; or

(4) Failure of the holder of exclusive approval to assure a sufficient quantity of the drug under section 527 of the act and § 316.36.

(b) Orphan-drug exclusive approval protects only the approved indication or use of a designated drug. If such approval is limited to only particular indication(s) or uses(s) within the rare disease or condition for which the drug was designated, FDA may later approve the drug for additional indication(s) or uses(s) within the rare disease or condition not protected by the exclusive approval. If the sponsor who obtains approval for these new indication(s) or uses(s) has orphan-drug designation for the drug for the rare disease or condition, FDA will recognize a new orphan-drug exclusive approval for these new (not previously approved) indication(s) or use(s) from the date of approval of the drug for such new indication(s) or use(s).

(c) If a sponsor's marketing application for a drug product is determined not to be approvable because approval is barred under section 527 of the Federal Food, Drug, and Cosmetic Act until the expiration of the period of exclusive marketing of another drug,